

Annual Report 2022



President's Report

The Global Porphyria Advocacy Coalition's (GPAC's) first official meeting was in Milan 3 years ago, at which point the first Executive Board was elected. At our last AGM (November 2021), Peder Sorenson (from Denmark) resigned as Treasurer and Marco Mo (from Canada) was voted as GPAC's new Treasurer. Over the last 12 months, GPAC have met regularly via Zoom to continue to establish GPAC's infrastructure. The rare disease community and GPAC have been impacted over the last three years in terms of networking opportunities and time to progress with GPAC aims, as a result of Covid/pandemic. We are very excited to be holding our 2022 AGM in Sofia, Bulgaria on Saturday, 4th September, where we will meet with many of our porphyria friends and colleagues as part of the biennial International Congress on Porphyrins and Porphyrin.

Progress to date

Meetings, connections and fostering global relationships: The GPAC Executive Board met virtually on 6 separate occasions to move actions forward for GPAC (20+ hours). The President and Vice-President met to discuss aims, strategic direction and tasks on at least 12 occasions (18+ hours). The President met on more than 15 individual occasions with various patient organisation leaders (12+ hours). The Executive also met with pharmaceutical/industry representatives on more than 6 occasions, resulting in a patient talk at one of our Member Meetings in May from Disc Medicine and planned talks/updates at our Global Gathering in Sofia from Modern Inc., Disc Medicine and Alnylam Pharmaceuticals. These connections for our rare community are invaluable in fostering change around trials and end-points as well as research conducted into the porphyrias, including quality of life studies. At our AGM Meeting last November, Professor Jean-Charles Deybach kindly delivered a very well-received pre-recorded presentation on new developments within the field of porphyria. GPAC Members meet virtually on 2 further occasions (7-8 hours), with representation from between 9 and 12 organisations. We are delighted to see that many organisations are including others from their group on the calls which is further engaging the community and allowing us to have a greater impact within the porphyria field. GPAC also represented the global porphyria community at an AHP Global Advisory Board with Alnylam, alongside other global porphyria leaders – an honorarium for which was donated to GPAC.

GPAC's Executives: In the last 12 months, GPAC has continued developing its infrastructure to enable they succeeding Executive Team to continue to move GPAC forward. GPAC managed to open a Global Community Bank Account with Barclays earlier this year, which resulted in various pledged donations being received from the APF, the BPA and Alnylam.

GPAC's Working Groups have focused on a number of projects which have evolved based on need/importance. In April, the **Awareness WG** delivered a wonderful Porphyria Awareness Week campaign – the theme 'We are porphyria together' was received well and observed as a great coming together of the porphyria patient community. The focus on the Friday of 'Purple for Porphyria' was visually impactful across social media and will be considered for inclusion moving forward.

Two new Working Groups emerged in response to needs in 2022. The **Cross-Border Connections WG** emerged from the desire to offer more support directly to patients, especially in countries where the communities are still small. The group held their first Global EPP Connect event, for people of all languages and ages. The event was a great success with around 25 people joining – a number of which had never met anyone with EPP before. This WG plans to hold another Global Connect event later this year, aimed at the Acute Porphyrias. GPAC will spend longer advertising and also focus more on native languages to generate the best discussions and accessibility for all.

More recently, in response to some very negative portrayals of porphyria in TV/streaming channels, a new **Media Working Group** has formed. The group is already active, responding to a specific TV programme on Netflix, as well as some other shows that are looking for storylines. The WG aims to develop strong responses and a general statement that can be endorsed by GPAC, patient groups and by the clinician networks. Thus giving a balanced voice for patients witnessing these negative portrayals. Further, the aim is to foster good relations that may lead to excellent portrayals of the porphyrias rather than sensationalised/inaccurate shows that are very upsetting to the porphyria patient community.

The **Treatments and Research Working Group** has provided regular updates at meetings which has generated much discussion and awareness of the state of trials/drug development and reimbursement across many countries. The German EPP group were delighted to report, earlier this year that Scenesse will be automatically renewed every year without the need for renewing/review. Scenesse is also available in Israel – congratulations to all involved in both of these achievements! Givlaari is available to patients in Poland too – again creating a step-change in the treatments available to patients. The APF Global Program continues to support numerous fledgling porphyria patient groups that GPAC hopes will become Members and support in due course.

Research and GPAC: Over the last year or so, various global patient representative from the GPAC community have been involved in the *Porphyria Worldwide Patient Experience Research (POWER) study*. Contributing to the design of the study, promoting participation amongst the global community and, over the last 12 months, being included within authorship for journal and conference submissions. The full manuscript was published in the Journal of Inherited Metabolic Diseases, it has since been presented with a different focus at 5 other conferences, and endorsed at further 5 conferences. This piece of work is a wonderful demonstration of the collaboration between industry, patient representatives and the patients themselves.

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2 New Working Groups

60+ Hours of collaborative working

3 New actively involved groups

Challenges faced: GPAC is still a very new organisation and we're still finding ways to work in a collaborative and inclusive manner. At our February 2022 Meeting the Vice-President led an interactive session aimed at developing a Charter for Working Together. The information gathered will be built into a Charter that we will ask all GPAC Members to respect and commit to. Alongside this, the Executives have been working on a Membership and Joining Procedure as well as a general Information Pack to support the work GPAC does and will do in the future.

Resource continues to be a huge problem and has resulted in a delay to finalising Membership processes and the Election of a new set of Executives to GPAC. A number of the Executives wish to step down/change roles and new enthusiasm and tenacity is needed within GPAC. The process for the Election of Officers will be presented, alongside a Membership Process (whilst in Sofia), once agreed a planned Extraordinary General Meeting (EGM) will be scheduled for early 2023.

Strategic aims for 2022

GPAC has many aims for the next 12 months. The Executive Committee will continue to **formalise GPAC's official infrastructure** over the coming weeks (including establishing bylaws, policies and procedures, roles and responsibilities, Member joining protocols as well as a membership fee collection processes), by the end of January 2023. To ensure all is ready for the EGM and to allow for **Elections** and a strong transfer of skilled individuals to a **New Executive Team**.

GPAC has plans to update the **website** – offer of assistance has come from the UPA and it may be that a WG could take this on as a project, to share the work. And, there has been an offer of help to register GPAC for **Office365** by a member of the BPA. The Working Groups will continue to evolve by setting tangible and clear aims and objectives (and roles and responsibilities) that can be reflected upon at our next AGM in 2023. As part of this, GPAC needs to evolve to ensure that the Executives are not overstretched and that the entity remains sustainable now and in the future.

It is hoped that GPAC will start working more with a **Buddy System** to ensure that New Groups are identified and better supported by the Members Organisations. **Translation services** will also be investigated as a real option to increase accessibility to our meetings and the community – it may be necessary to apply for a specific budget for this.

GPAC hopes to be involved in **future research** with other industry representatives so the burden of porphyria is more widely understood and to further validate to the existing patients that they are not alone in the impact their porphyria has on them and their families.

GPAC would also like to acknowledge and thank a few key people who have been involved in a number of other research publications. A new **Research and Information WG** will be starting work shortly on an online resource that will store papers such as these, to make it easy for the GPAC community to access them.

Treasurer's Report

In March 2022, we had confirmation that GPAC's Community Account with Barclays Bank was officially open. Soon after which GPAC contacted the APF, the BPA and Alnylam regarding the staff donation from 2021 to GPAC. All funds were very gratefully received by GPAC, as follows.

GPAC Income 2022		GBP	GBP
Date received		Income	
29/03/2022	BPA – initial donation 500 GBP	£500.00	
17/05/2022	APF – initial donation 1,000 USD	£780.47	
10/06/2022	Alnylam staff donation 5,000 USD*	£3,981.16	
09/08/2022	Honorarium payment for ad-board Alnylam	675.00	
01/09/2022	Alnylam Sponsorship for AGM & Global Gathering	£500	
Total Income		£6436.63	£6436.63
Expenditure in 2022			
31/08/2022	Expenses to S.Burrell of £713.95 (All paid and reimbursed to S.Burrell - Webdomain 2020-2022, Zoom, Epnet Associate Membership of 400 Euros 2019-2022)	£713.95	
Total expenditure		- £713.95	- £713.95
Gross profit			£5,722.68

Please Note: On GPAC's projected income on the last financial report, there was a note to reimburse 30 Euros to Danny De Roode. However, Danny kindly decided to gift/donate this cost to GPAC, so has been removed from the accounts. *Also note, the Alnylam staff donation of 5,000 USD, not the 3,000 USD initially documented.

Income and Expenditure Account for the period 11 November 2021 - 10 November 2022
Global Porphyria Advocacy Coalition
CIO Reg No: 1189694

	GBP £	GBP £
Income	6867.31	6867.31
 Less Expenditure		
Software subscriptions (Zoom)	143.88	
Website and telephone	213.07	
Memberships (professional)	357	
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	713.95	713.95
 Income/expenditure		<hr/>
		6153.36